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## Economic evaluation of lung transplantation

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## Summary

During the last decades, development and dissemination of new medical interventions occurred rapidly. Consequently, the demand for as well as the costs associated with health care increased strongly. Since then, particular health care decision makers have tried to control those health care expenditures. In order to make justifying choices possible a growing need for broad evaluations of health care interventions arose, and Medical Technology Assessment (MTA) and efficiency studies have emerged as new research disciplines. While the scope of the first type of research is somewhat broader, both types include an economic evaluation, in which the costs and health consequences of two or more alternatives are assessed and compared.

In the first part of this thesis an introduction to economic evaluation was given; the consequences of performing an economic evaluation alongside a clinical trial (Chapter 2) and the basic concepts of economic evaluation (Chapter 3) were addressed. As economic evaluation is always about both health consequences and costs, the consequence of adding an economic evaluation to a clinical trial is always that besides medical outcomes, costs have to be assessed. However, other adjustments in the design may also be necessary, such as, for instance, the addition of quality of life valuations (utilities) and the extension of the time horizon. Collaboration between health economists and clinical researchers is, therefore, advisable from the start of the study design. In this way, one can work on an integrated design, which is suitable to answer both clinical and economic research questions.

In the second (central) part of this thesis the methods and results of the MTA of the Dutch lung transplantation programme were described. This study, an example of a trial including an economic evaluation, was initiated by the Dutch National Health Insurance Board, to provide this board with information about the clinical effectiveness, quality of life, costs, cost-effectiveness, and the need for and supply of donor lungs. For reason of comparison, the study was set up according to the protocols of the assessments of the Dutch liver and heart transplantation programmes. Selection criteria for lung transplant recipients in the transplantation programme were the presence of irreversible, progressively disabling end-stage pulmonary or cardiopulmonary disease. The following diagnoses were distinguished: emphysema, cystic fibrosis, pulmonary hypertension, pulmonary fibrosis, and bronchiectasis.

The main focus of this thesis was on the economic evaluation, which was part of the MTA of lung transplantation. In this economic evaluation, the costs and effects (= health consequences with regard to survival and quality of life) for patients enrolled in the Dutch lung transplantation programme were compared to the costs and effects in the situation without a programme. For such a comparison, in general,

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a randomised clinical trial is considered as the most optimal study design. However, in view of the expected positive effects of lung transplantation on survival, it was considered unethical to perform a randomised clinical trial. Therefore, all suitable patients were admitted to the transplantation programme. Consequently, only costs and effects with the programme were observed and costs and effects in the situation without the programme (in which patients receive a usual treatment for their lung disease until death) were estimated on the base of pre-transplantation evidence.

The results of the MTA of lung transplantation revealed that, on average, patients live longer if they are transplanted than compared to if they continue to receive a conventional treatment. The average survival after transplantation was estimated at 7.4 years. If not transplanted, on average, patients would only have lived for 3.0 years, which means that (on average) patients live 4.4 years longer if they get a lung transplantation.

The assessment of the quality of life before and after lung transplantation (Chapter 5) showed that lung transplantation leads to a substantial improvement in the health-related quality of life. Before transplantation, patients report major restrictions on the dimensions mobility and energy, when the Nottingham Health Profile was used. In addition, they experienced a low level of well-being and depressive symptoms, difficulties in performing activities of daily life (e.g. dressing, washing, and going up and down stairs), and a low ability to take care of themselves. About 4 months after transplantation, mobility, energy, sleep, activities of daily life dependency level, and dyspnea were particularly positively affected by the lung transplantation. These improvements were maintained in the following 15 months.

In contrast to the positive effects of lung transplantation on survival and quality of life, considerable costs were induced by the lung transplantation programme. A comparison between the costs in the situation with and without a transplantation programme (Chapter 4) showed that the additional costs induced by lung transplantation are about half a million Dutch guilders per transplanted patient. In this amount, besides costs induced within health care, also costs induced by the patients and their families and production losses are included.

To address the cost-effectiveness of the Dutch lung transplantation programme, the additional costs of lung transplantation were related to the gains in survival and quality of life (Chapter 6). Main outcome was that it costs Dfl.152.000 to let one patient live one year longer by receiving a lung transplant. If the survival was adjusted for the quality of life, the outcome was an amount of Dfl. 120.000 per Quality Adjusted Life Year. From scenario analyses (Chapter 7) it became clear that, if no changes in the transplantation programme are made, those ratios will become even higher in the future.

The results of lung transplantation were compared to the results of the Dutch heart and liver transplantation programmes (Chapter 9). This analysis showed that the cost-effectiveness of lung transplantation is unfavourable compared to those of

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the Dutch heart and liver transplantation programmes. There are two main reasons for this unfavourable cost-effectiveness. First of all, the survival gain is relatively small for lung transplant recipients, due to a relatively low survival after transplantation and a relatively long survival of lung transplant patients on the waiting list. Survival after transplantation is relatively low, because of (i) the high risk of acute rejection, (ii) the high risk of infection and (iii) the invariably high risk of bronchiolitis obliterans syndrome (BOS), a chronic transplant dysfunction. Survival on the waiting list is relatively high, because of a large proportion of patients with emphysema (usually with a long survival on the waiting list) and the difficulty to determine the optimal moment to place a patient on the waiting list. Moreover, a bias towards premature placement of patients on the waiting list may be caused by the shortage of donor lungs and the allocation algorithm (first in, first out) of the transplant organisation. A second reason for the unfavourable cost-effectiveness of lung transplantation is the high follow-up costs of lung transplant recipients compared to heart and liver transplant recipients. High follow-up costs are caused by the infection and rejection problems of the patients, including the development of BOS by a large part of the patients. From additional analyses (Chapter 10) it was showed that the patients who developed BOS during follow-up induced considerable extra costs compared to the patients who did not develop BOS.

Besides a comparison with other Dutch transplantation programmes, the results of the Dutch lung transplantation study were compared to the results of an American study about the cost-effectiveness of lung transplantation. This American study showed much favourable outcomes with regard to costs and cost-effectiveness than the Dutch study. Nevertheless, the methods used in this study were highly questionable; several relevant costs were excluded (e.g. the costs of follow-up) and no comparison with the situation without a transplantation programme was made. By an extrapolation it was shown, that higher costs and cost-effectiveness ratios are more likely for the American situation.

Finally, it was investigated how the cost-effectiveness of lung transplantation may be improved in the future (Chapter 9). Therefore, besides the comparison with the other two Dutch transplantation programmes, sensitivity analyses were performed. Those analyses revealed that, to improve the cost-effectiveness of lung transplantation substantially, an improvement in the survival after transplantation or a decrease in the follow-up costs is necessary. Other possibilities, such as for instance a change in the patient inclusion criteria (e.g. the exclusion of patients with the smallest gain in survival and/or quality of life), are examined in the efficiency study of lung transplantation, which is still in progress.

In the meantime, the minister of Health Affairs decided, that because of the proven effectiveness of lung transplantation, this procedure has to be included into the Dutch benefit package.